



4164-01-P

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2019-N-1468]

Characterizing the Food and Drug Administration's Approach to Benefit-Risk Assessment

Throughout the Medical Product Life Cycle; Public Meeting; Request for Comments

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice of public meeting; request for comments.

SUMMARY: The Food and Drug Administration (FDA, the Agency, or we) is announcing the following public meeting entitled "Characterizing FDA's Approach to Benefit-Risk Assessment Throughout the Medical Product Life Cycle" and an opportunity for public comment. The meeting will be convened by Duke University's Robert J. Margolis, MD, Center for Health Policy (Duke-Margolis) and supported by a cooperative agreement with FDA. The meeting is intended to gather industry, patient, researcher, and other stakeholder input on applying FDA's Benefit-Risk Framework throughout the human drug lifecycle and best approaches to communicating FDA's benefit-risk assessment. Input from this meeting will support development of a draft guidance on benefit-risk assessment for new drugs and biologics and result in a publicly available summary report from Duke-Margolis. This meeting is intended to meet an FDA commitment included in the sixth authorization of the Prescription Drug User Fee Amendments of 2017 (PDUFA VI).

DATES: The public meeting will be held on May 16, 2019, from 9 a.m. to 5 p.m. Submit either electronic or written comments on this public meeting by June 17, 2019. See the

SUPPLEMENTARY INFORMATION section for registration date and information.

ADDRESSES: The public meeting will be held at the Tommy Douglas Conference Center, 10000 New Hampshire Ave., Silver Spring, MD 20903. For information on the public meeting location please see <https://www.tommydouglascenter.com/>.

You may submit comments as follows. Please note that late, untimely filed comments will not be considered. Electronic comments must be submitted on or before June 17, 2019. The <https://www.regulations.gov> electronic filing system will accept comments until 11:59 p.m. Eastern Time at the end of June 17, 2019. Comments received by mail/hand delivery/courier (for written/paper submissions) will be considered timely if they are postmarked or the delivery service acceptance receipt is on or before that date.

#### *Electronic Submissions*

Submit electronic comments in the following way:

- Federal eRulemaking Portal: <https://www.regulations.gov>. Follow the instructions for submitting comments. Comments submitted electronically, including attachments, to <https://www.regulations.gov> will be posted to the docket unchanged. Because your comment will be made public, you are solely responsible for ensuring that your comment does not include any confidential information that you or a third party may not wish to be posted, such as medical information, your or anyone else's Social Security number, or confidential business information, such as a manufacturing process. Please note that if you include your name, contact information, or other information that identifies you in the body of your comments, that information will be posted on <https://www.regulations.gov>.

- If you want to submit a comment with confidential information that you do not wish to be made available to the public, submit the comment as a written/paper submission and in the manner detailed (see “Written/Paper Submissions” and “Instructions”).

### *Written/Paper Submissions*

Submit written/paper submissions as follows:

- Mail/Hand delivery/Courier (for written/paper submissions): Dockets Management Staff (HFA-305), Food and Drug Administration, 5630 Fishers Lane, Rm. 1061, Rockville, MD 20852.
- For written/paper comments submitted to the Dockets Management Staff, FDA will post your comment, as well as any attachments, except for information submitted, marked and identified, as confidential, if submitted as detailed in “Instructions.”

*Instructions:* All submissions received must include the Docket No. FDA-2019-N-1468 for “Characterizing FDA’s Approach to Benefit-Risk Assessment Throughout the Medical Product Life Cycle; Public Meeting; Request for Comments.” Received comments, those filed in a timely manner (see ADDRESSES), will be placed in the docket and, except for those submitted as “Confidential Submissions,” publicly viewable at <https://www.regulations.gov> or at the Dockets Management Staff between 9 a.m. and 4 p.m., Monday through Friday.

- Confidential Submissions--To submit a comment with confidential information that you do not wish to be made publicly available, submit your comments only as a written/paper submission. You should submit two copies total. One copy will include the information you claim to be confidential with a heading or cover note that states “THIS DOCUMENT CONTAINS CONFIDENTIAL INFORMATION.” The Agency will review this copy, including the claimed confidential information, in its

consideration of comments. The second copy, which will have the claimed confidential information redacted/blacked out, will be available for public viewing and posted on <https://www.regulations.gov>. Submit both copies to the Dockets Management Staff. If you do not wish your name and contact information to be made publicly available, you can provide this information on the cover sheet and not in the body of your comments and you must identify this information as “confidential.” Any information marked as “confidential” will not be disclosed except in accordance with 21 CFR 10.20 and other applicable disclosure law. For more information about FDA’s posting of comments to public dockets, see 80 FR 56469, September 18, 2015, or access the information at: <https://www.gpo.gov/fdsys/pkg/FR-2015-09-18/pdf/2015-23389.pdf>.

*Docket:* For access to the docket to read background documents or the electronic and written/paper comments received, go to <https://www.regulations.gov> and insert the docket number, found in brackets in the heading of this document, into the “Search” box and follow the prompts and/or go to the Dockets Management Staff, 5630 Fishers Lane, Rm. 1061, Rockville, MD 20852.

FOR FURTHER INFORMATION CONTACT: Graham Thompson, Center for Drug Evaluation and Research, Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 51, Rm. 1146, Silver Spring, MD 20993-0002, 301-796-5003, Fax: 301-847-8443, [Graham.Thompson@fda.hhs.gov](mailto:Graham.Thompson@fda.hhs.gov); or Stephen Ripley, Center for Biologics Evaluation and Research, Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 71, Rm. 7301, Silver Spring, MD 20993-0002, 240-402-7911, [Stephen.Ripley@fda.hhs.gov](mailto:Stephen.Ripley@fda.hhs.gov).

SUPPLEMENTARY INFORMATION:

## I. Background

This public meeting is intended to satisfy a commitment included in PDUFA VI. This PDUFA reauthorization is part of the FDA Reauthorization Act of 2017 signed by the President on August 18, 2017. The complete set of performance goals and procedures documented in the PDUFA Reauthorization Performance Goals and Procedures Fiscal Years 2018 through 2022 (Goals Letter) is available at <https://www.fda.gov/downloads/ForIndustry/UserFees/PrescriptionDrugUserFee/UCM511438.pdf>. These goals were developed in consultation with patient and consumer advocates, healthcare professionals, and other public stakeholders as part of negotiations with industry. Section I.J.2 of the Goals Letter, “Enhancing Benefit-Risk Assessment in Regulatory Decision-Making,” outlines the commitment for FDA to convene and/or participate in a public meeting to gather stakeholder input on key topics relating to FDA’s benefit-risk assessment.

## II. Topics for Discussion at the Public Meeting

This meeting will provide FDA the opportunity to gather input from stakeholders on their experiences and perspectives regarding FDA’s benefit-risk assessment. Input from this meeting will support development of the draft guidance on benefit-risk assessment for new drugs and biologics as outlined in Section I.J.2 of the Goals Letter, which FDA intends to issue by the end of June 2020. The meeting will allow participants (including industry, patients, researchers, and other stakeholders) to provide input on key topics, including the application of FDA’s Benefit-Risk Framework throughout the human drug lifecycle and information that sponsors may develop or collect at the various stages of drug development that can inform the benefit-risk assessment and related regulatory decisions. This includes consideration of how relevant patient experience data and related information may inform the benefit-risk assessment. In addition, the

meeting will consider appropriate approaches to communicate to the public FDA's thinking regarding a product's benefit-risk assessment.

For more information on meeting topics and discussion questions, visit <https://healthpolicy.duke.edu/events/benefit-risk-framework-public-workshop>. FDA will publish a background document outlining the topic areas that FDA plans to address in the draft guidance to this site approximately 2 weeks before the meeting date. FDA will also post the agenda and other meeting materials to this site approximately 5 business days before the meeting.

The format of the meeting will consist of a series of presentations, panel discussions, and audience Q&As. In addition to input generated through this public meeting, FDA is interested in receiving input on the planned draft guidance through written comments, which can be submitted to the public docket (see ADDRESSES).

### III. Participating in the Public Meeting

*Registration:* To register for the public meeting, please visit the following website: <https://events.r20.constantcontact.com/register/eventReg?oeidk=a07eg01qxxd45281872&oseq=&c=&ch>. Please register by May 10, 2019. If you are unable to attend the meeting in person, you can register to view a live webcast of the meeting. You will be asked to indicate in your registration if you plan to attend in person or via the webcast. Please provide complete contact information for each attendee, including name, title, affiliation, address, email, and telephone.

Registration is free and based on space availability, with priority given to early registrants. Persons interested in attending this public meeting must register by May 10, 2019, 11:59 p.m. Eastern Time. Early registration is recommended because seating is limited; therefore, FDA may limit the number of participants from each organization. Registrants will receive confirmation once they have been accepted. If time and space permit, onsite registration

on the day of the public meeting will be provided beginning at 8 a.m. We will let registrants know if registration closes before the day of the public meeting.

If you need special accommodations due to a disability, please contact Graham Thompson no later than May 10, 2019, 11:59 p.m. Eastern Time.

*Open Public Comment:* There will be time allotted during the meeting for open public comment. Sign-up for this session will be on a first-come, first-served basis on the day of the meeting. Individuals and organizations with common interests are urged to consolidate or coordinate and request time for a joint presentation. No commercial or promotional material will be permitted to be presented or distributed at the public meeting.

*Streaming Webcast of the Public Meeting:* This public meeting will also be webcast. Please register for the webcast by visiting <https://events.r20.constantcontact.com/register/eventReg?oeidk=a07eg01qxxd45281872&oseq=&c=&ch>.

FDA has verified the website addresses in this document as of the date this document publishes in the *Federal Register*, but websites are subject to change over time.

*Transcripts:* Please be advised that as soon as a transcript of the public meeting is available, it will be accessible at <https://www.regulations.gov>. It also may be viewed at the Dockets Management Staff (see ADDRESSES).

Dated: April 18, 2019.

Lowell J. Schiller,

Principal Associate Commissioner for Policy.

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